

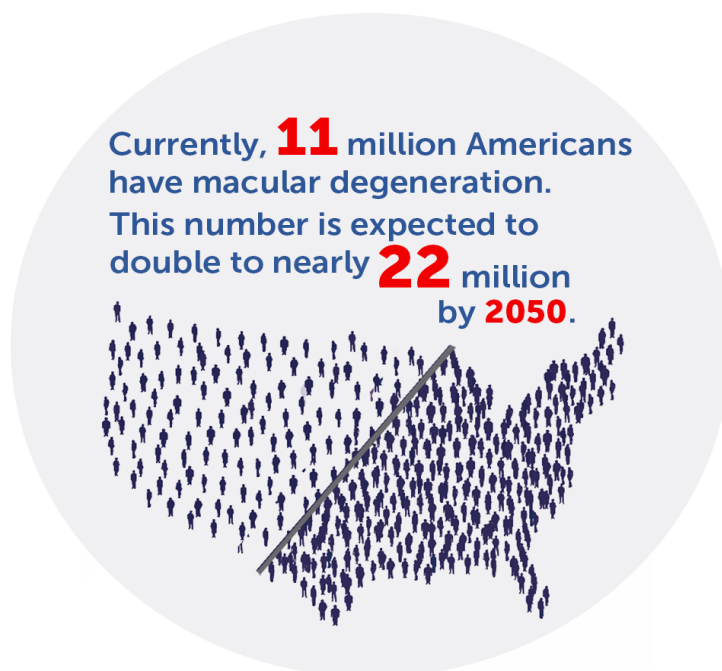
Macular Degeneration Research Current Grantees

Advanced forms of age-related macular degeneration (AMD) are a leading cause of vision loss and irreversible blindness in Americans age 60 years and older, as well as throughout the world. Much more work is needed to develop effective treatments and cures.

With the support of our donors, Macular Degeneration Research (MDR) has awarded more than \$24 million to fund research projects on the causes and potential prevention, treatment, and cure of this disease.

To defeat macular degeneration, MDR invests in urgently needed research involving numerous avenues that offer the promise of ending this disease. We fund a broad array of scientific approaches, encompassing innovative projects falling into the following categories:

- Understanding Early AMD
- Drusen Formation and the Immune Response
- Genes and AMD
- How Diet and Nutrition Affect AMD Risk
- Advancing New AMD Treatments and Technology
- Special Focus on Geographic Atrophy



Understanding Early AMD

Macular degeneration is a disease linked to not just one, but many, causes. Foremost are changes in the eye that happen with age—the strongest risk factor. It is generally thought that age-related macular degeneration (AMD) begins in the retinal pigment epithelium (RPE), a layer of cells next to the retina, whose job is to transport molecules in and out to nourish the retina and dispose of waste. The RPE's ability to do its job can be compromised by age, oxidative stress, inflammation, and other factors causing the immune system to kick in and overreact. Grantees are looking at additional problems, like lipid imbalance and declines in cellular energy production, as possible triggers to AMD. The scientific exploration that MDR is funding will expand our understanding and open new and earlier treatment avenues.



Maria Valeria Canto-Soler, PhD (7/1/16 - 6/30/18)
Wilmer Eye Institute, Johns Hopkins University, Baltimore, MD

A New Model of a Human Retina in a Dish to Study AMD

"Our goal is to develop the first "human retina in a dish" model...to provide a unique biological system to investigate the initial triggers leading to AMD and to develop treatments to stop its progress."

www.brightfocus.org/grant/M2016119

Recipient of The Helen Juanita Reed Award



Yan Chen, PhD (7/1/17 - 6/30/19)
The University of Texas Medical Branch at Galveston

Metabolic Pathways of the Retina in Health and AMD

"In this project we will study the mechanisms of energy production and regulation, in both healthy and diseased eyes, particularly those with AMD."

www.brightfocus.org/grant/M2017186



Noriko Esumi, MD, PhD (7/1/15 - 6/30/18)
Johns Hopkins University, Baltimore, MD

Resistance to Oxidative Stress: A New Strategy for AMD

"The goal of this project is to develop new strategies for prevention and treatment of AMD, more specifically to test a molecule that promotes stress resistance and longevity on the survival and integrity of the RPE, a critical cell for AMD."

www.brightfocus.org/grant/M2015220

This grant is made possible by bequests from the Helen Juanita Reed Irrevocable Trust and the Helen Juanita Reed Charitable Remainder Unitrust.

Recipient of The Helen Juanita Reed Memorial Award.



Robyn Guymer, PhD (7/1/16 - 6/30/18)
Centre for Eye Research Australia, University of Melbourne

Too Much Debris as a Cause of AMD

"The goal of our project is to provide a novel explanation for the accumulation of debris that contributes to AMD."

www.brightfocus.org/grant/M2016061

Recipient of The Carolyn K. McGillvray Memorial Award



Ernesto Moreira, MD (7/1/15 - 6/30/18)
Medical University of South Carolina, Charleston

Using Patient-Derived Stem Cells as a New Model to Study Disease Mechanisms in Age-related Macular Degeneration

"In this project we are investigating how toxins derived from cigarette smoke lead to RPE cell damage through the activation of the immune complement system."

www.brightfocus.org/grant/M2015356



Sarah McFarlane, PhD (7/1/17 - 6/30/19)
University of Calgary, Canada

Aberrant Blood Vessel Growth in AMD: A New Animal Model

"We are developing a genetic animal model where we can rapidly identify novel, safe and effective drugs for the treatment of neovascular AMD."

www.brightfocus.org/grant/M2017002



Ruchira Singh, PhD (7/1/15 - 6/30/18)
University of Rochester Medical Center, New York

Understanding the Role of Different Cells in the Eye that Are Affected in AMD

"The overall goal of this project is to understand the role of different cells in the eye that are affected in macular degeneration, and which of them initiate disease processes; culminating in visual dysfunction."

www.brightfocus.org/grant/M2015267



Michael Paulaitis, PhD (7/1/17 - 6/30/19)
Johns Hopkins University, Baltimore, MD

MicroRNAs and Mitochondrial Dysfunction in AMD

“Our overall goal is to test the novel concept that a recently discovered class of small non-coding RNAs, so-called microRNAs, which are encapsulated in nanometer-sized vesicles secreted by RPE cells, can predict the status of mitochondrial function in these cells and influence mitochondrial function in neighboring RPE cells through vesicle-mediated intercellular transfer of the microRNAs.”

www.brightfocus.org/grant/M2017060

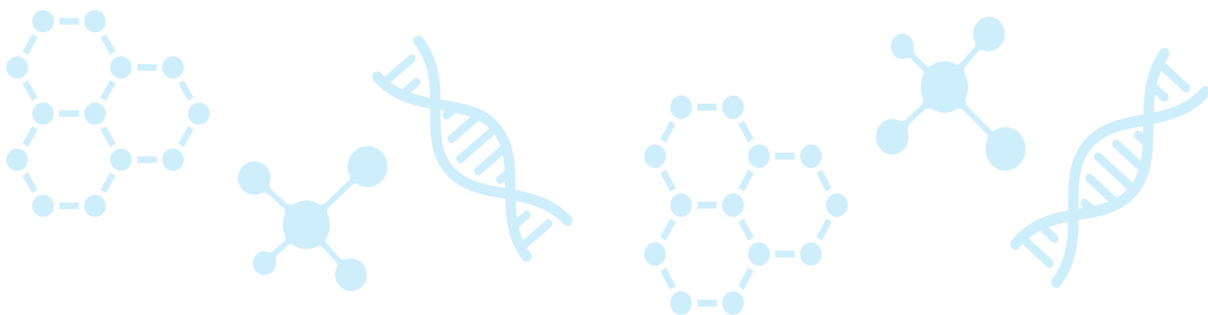


Claudio Punzo, PhD (7/1/17 - 6/30/19)
University of Massachusetts School of Medicine, Worcester

Role of the Light-sensing Photoreceptor Cells in AMD

“Our hypothesis is that AMD is caused by photoreceptors, more precisely, by age-related metabolic adaptations in photoreceptors. To test this hypothesis we have generated a new mouse model with altered cell metabolism in photoreceptors. This mouse model recapitulates many features of human AMD.”

www.brightfocus.org/grant/M2017071



Drusen Formation and the Immune Response

As the eye ages, it becomes less efficient at removing waste, causing the immune system to work overtime. Fatty deposits known as drusen may collect under the retina and trigger an immune response. In fact, when spotted on a comprehensive eye exam, drusen often are the first sign of disease. A stiffening of blood vessels may contribute further. Inflammation and the immune response are like double-edged swords: sometimes helpful and sometimes harmful. Ultimately, an out-of-control immune response may reach a tipping point and damage cells in the macula or central part of the eye, that provides the closely-focused vision. Thus, researchers are focusing on specific aspects of the immune response, including numerous inflammatory factors, and the eye's own built-in defense molecules, called microglia, to learn exactly how they interact and participate in AMD.



Marie Burns, PhD (7/1/16 - 6/30/18)

University of California, Davis

Window to Health: New Ways to Detect the First Signs of Cell Sickness in the Eye

"One big-picture goal of our research is to identify cells in distress and to heal them before they die and before patients lose their sight."

www.brightfocus.org/grant/M2015379

This grant is made possible in part by a grant from the Ivan Bowen Family Foundation.



Patrick Daugherty, PhD (7/1/16 - 6/30/18)

University of California, Santa Barbara

Characterization of Circulating Antibodies Specific to AMD

"The objective of this study is to characterize the changes in the immune response as individuals develop AMD. The end result of this effort will be to develop diagnostics for early detection."

www.brightfocus.org/grant/M2016219



Sarah Doyle, PhD (7/1/16 - 6/30/18)

Trinity College Dublin, Ireland

Investigating How Loss of an "Off Switch" for Inflammation Contributes to AMD

"AMD has elements that indicate that the inflammatory response is uncontrolled and persistent when low-level inflammation is observed. Our research question asks whether this active process of switching off the inflammatory response is lost in people with AMD."

www.brightfocus.org/grant/M2016030



Malia Edwards, PhD (7/1/16 - 6/30/18)

Wilmer Eye Institute, Johns Hopkins University, Baltimore, MD

A Study of Why Retinal Support Cells, Called Glia, Exit the Retina in AMD

"The goal of my research is to identify glial cell changes in AMD and determine how these may affect AMD progression and treatment."

www.brightfocus.org/grant/M2016198

This grant was made possible in part by a bequest from the Robert H. McLaren Trust.



Francesco Giorgianni, PhD (7/1/16 - 6/30/18)

University of Tennessee Health Science Center, Memphis

Basic and Clinical Studies to Understand the Role of the CD5L/AIM Protein in AMD

"The main objective of our research project is to reveal the cellular and molecular mechanisms involved in AMD by studying two key pathological events that characterize AMD: the accumulation of drusen deposits and the death of retinal pigment epithelium cells (RPE)."

www.brightfocus.org/grant/M2016068

This grant is made possible in part by a bequest from the Stuart Blydenburgh Trust.



Kaustabh Ghosh, PhD (7/1/16 - 6/30/18)

University of California, Riverside

Understanding the Role of Increased Vessel Stiffness in Cell Death Associated with AMD

"In this project, we are investigating the hypothesis that aging leads to stiffening of blood vessels in the eye that, in turn, exacerbates the pathogenesis of AMD by causing inflammation-mediated vascular degeneration."

www.brightfocus.org/grant/M2016161



Cristhian Ildefonso, PhD (7/1/17 - 6/30/19)

University of Florida, Gainesville

Exploring the Role of Inflammation in AMD

"My research goal is to improve a mouse model of this disease by introducing pro-inflammatory cues, and to experiment with a method of decreasing inflammation in the eye to test the idea that localized control of inflammation could help protect vision."

www.brightfocus.org/grant/M2017126

Recipient of The Helen Juanita Reed Award



Goldis Malek, PhD (7/1/15 - 12/31/18)
Duke University, Durham, NC

The Role of an Immune Cell Attractant in a Blinding Disease

"The experiments in this study are designed to address the following question: What is the role of a specific factor called osteopontin that may be responsible for recruiting immune cells to the eye in the development and progression of AMD?"

www.brightfocus.org/grant/M2015421

This grant is made possible by a gift made in memory of Carolyn K. McGillvray. Recipient of The Carolyn K. McGillvray Award.



Daniel Saban, PhD (7/1/17 - 6/30/19)
Duke University Eye Center, Durham, NC

Targeting Immune Cells in AMD

"Our grant project seeks to identify the specific immune cell type that contributes to vision loss, and to devise a strategy that neutralizes such cells to ultimately help preserve vision in patients suffering from age-related macular degeneration."

www.brightfocus.org/grant/M2017183

Genes and AMD

Most forms of macular degeneration are not linked to any single genetic mutation. Instead, susceptibility to AMD is scattered over a number of small irregularities of genes called single nucleotide polymorphisms (SNPs). SNPs may arise spontaneously or be inherited, and their impact is tempered by other factors, such as age, overall health and nutrition, and exposure to smoke, sunlight, and other toxins. Despite their relatively indirect influence, genes may be one way to lower the risk of AMD, if researchers can promote the survival and integrity of the RPE as it encounters oxidative stress from aging and other causes, and block or replace gene signals that trigger disease.



Paul Baird, PhD (7/1/16 - 6/30/18)
Centre for Eye Research Australia, The University of Melbourne

Identifying Gene Pathways in Late-Stage AMD

"This project will look at how different regions of our genetic background interact with other genetic regions and lead to AMD."

www.brightfocus.org/grant/M2016178



Astra Dinculescu, PhD (7/1/17 - 6/30/19)
University of Florida, Gainesville

Extracellular Deposits and Vision Loss in AMD

"Our goal is to understand the factors contributing to the formation of drusen deposits, in order to develop a strategy to eliminate them and preserve vision in AMD patients."

www.brightfocus.org/grant/M2017035



Stefanie Hauck, PhD (7/1/15 - 12/31/17)
Helmholtz Zentrum München GmbH, Neuherberg, Germany

Identification of Protein Complexes Binding to Genomic AMD Risk Variants

"As a result of this project, we expect to uncover novel pathways that are associated with AMD genetic risk factors and these may provide the basis for innovative preventive therapies."

www.brightfocus.org/grant/M2015370



Philippe Mourrain, PhD (7/1/17 - 6/30/19)
Stanford University, California

Zebrafish Provides Insights into New AMD-Associated Genetic Mutation

"This study will not only identify the actual impact of this non-coding human mutation relative to AMD, but also characterize how the evolutionarily conserved DNA region bearing the mutation in human regulates its surrounding genes, including COL8A1, in the eye."

www.brightfocus.org/grant/M2017209

This grant is made possible by support from the Nancy F. Seeley Trust in memory of Mildred F. Ferguson.



Patsy Nishina, PhD (7/1/17 - 6/30/19)
The Jackson Laboratory, Bar Harbor, ME

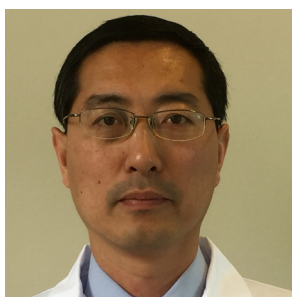
DNA Changes That May Lead to AMD and Other Vision Disease

"This project will establish a research pipeline to examine the effects of the extracellular matrix (ECM)-related risk alleles in cultured retinal pigmented epithelium (RPE) cells and select those alleles that cause the greatest effects to generate mouse models that will be distributed to the research community."

www.brightfocus.org/grant/M2017042

How Diet and Nutrition Affect AMD Risk

The current standard treatment for early-moderate dry AMD consists of eye vitamins that follow the AREDS [Age-Related Eye Disease Study] formula. However, there may be additional ways to lower risk, given how sensitive the eye is to nutritional intake and possible deficiencies. Carotenoids (molecules that give the bright red, yellow and orange color to fresh produce) are vital to macular health, and there may be ways to boost the body's uptake of this important nutrient. Vitamin C is being investigated to delay and/or prevent AMD progression. Our diets also may influence how our body responds to disease, and help shape a healthy immune response, by influencing the composition and function of the micro-organisms that live within our body (aka, our gut bacteria). The hope is that all these findings may be rapidly translated for the clinic.



Binxing Li, PhD (7/1/17 - 6/30/19)

Moran Eye Center, Salt Lake City, UT

Delivering Sight-saving Nutrients to the Retina in AMD

"Our goal is to understand the mechanism of scavenger receptor BI (SR-BI)-mediated macular carotenoid transport."

www.brightfocus.org/grant/M2017066



Trever McGill, PhD (7/1/17 - 6/30/19)

Oregon Health & Science University, Portland

Nutritional Factors in the Development of AMD

"The goal of this project is to determine whether being deprived of these nutrients has consequences for the development of AMD, and to determine the mechanisms by which this occurs."

www.brightfocus.org/grant/M2017073

Recipient of The Carolyn K. McGillvray Award



Sheldon Rowan, PhD (7/1/17 - 6/30/19)

Tufts University, Boston, MA

Importance of Gut Bacteria in A Model of AMD

"The goal of my proposal is to test whether the risk for developing AMD is based in part on our diet and nutrition, and relates to the composition and function of the micro-organisms that live within our guts, collectively known as the gut microbiome."

www.brightfocus.org/grant/M2017147

Recipient of The Elizabeth Anderson Award



Gaofeng Wang, PhD (7/1/17 - 6/30/19)

Miller School of Medicine, University of Miami, Coral Gables, FL

Using Vitamin C to Treat AMD

"This research aims to use vitamin C to inhibit the production of VEGF [vascular endothelial growth factor, a major factor promoting the growth of new blood vessels in the eye]... Successful completion of this project will help to develop an ascorbate treatment to delay and/or prevent AMD progression that is inexpensive and readily available for AMD patients."

www.brightfocus.org/grant/M2017081

Advancing New AMD Treatments and Technology

What will be the next "gold standard" treatment for macular degeneration? MDR is funding research into unique ways to protect the retinal pigmented epithelium (RPE) and retina at earlier stages, before damage to sight has occurred. These include drugs that enhance immune functioning and improve the eye's ability to clear lipids and other waste that might otherwise lead to inflammation in AMD, as well as neuroprotective drugs that help cells survive when they're under attack. Researchers are also looking at ways to diagnose AMD earlier and restore health to the aging eye by improving its metabolism, or efficiency at storing and using energy.



Petr Baronov, MD, PhD (7/1/16 - 6/30/18)

Schepens Eye Research Institute, Massachusetts Eye and Ear, Harvard Medical School, Boston, MA

A New Approach to Rescuing Photoreceptors from Death through Activation of Endogenous Neuroprotective Mechanisms

"We aim to identify small molecules that can induce endogenous growth factors in the sensory part of the eye, the retina. The results of our studies should lead to the development of novel, accessible and effective molecular therapies for retinal degeneration and other neurodegenerative disorders."

www.brightfocus.org/grant/M2016046



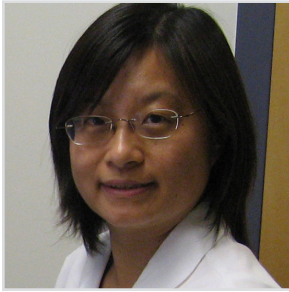
Daniel Chao, MD, PhD (7/1/17 - 6/30/19)

University of California, San Diego

Drug Discovery in a Zebrafish Model of Wet AMD

"The purpose of the grant is to develop zebrafish, a common model organism, as a model for drug discovery for macular degeneration."

www.brightfocus.org/grant/M2017034



Jing Chen, PhD (7/1/17 - 6/30/19)
Children's Hospital Boston, Harvard Medical School, Massachusetts

Protecting RPE and Photoreceptors in AMD

"Our work aims to investigate mechanistically the molecular processes through which dysregulation of factors controlling oxidative stress impairs RPE and photoreceptor cell metabolism and their survival in AMD."

www.brightfocus.org/grant/M2017161



Kip Connor, PhD (7/1/16 - 6/30/18)
Schepens Eye Research Institute, Massachusetts Eye and Ear, Harvard Medical School, Boston, MA

Lipid Regulators of AMD

"Our proposal has clear potential to lead to new therapeutic molecules, targets, and strategies for specifically inhibiting neovascular AMD, a leading cause of blindness, if left untreated, rapidly leads to substantial vision loss."

www.brightfocus.org/grant/M2016183

This grant is made possible in part by a bequest from the Estate of Robert J. Mac.



Rajendra Kumar-Singh, PhD (7/1/17 - 6/30/19)
Tufts University, Boston, MA

Developing a Gene Therapy Against Complement Factors in AMD

"The goal of our project is to determine the molecular factors responsible for pathology in AMD and to utilize that knowledge to develop therapies."

www.brightfocus.org/grant/M2017175



Marcelo Nociari, PhD (7/1/16 - 6/30/18)
Weill Medical College, Cornell University, New York

Identification of Novel Treatments for Macular Degeneration by Alleviating Endoplasmic Reticulum Stress

"We found a novel mechanism by which lipid-bisretinoids (LBs) kill RPE cells. In the current project, we propose to fully characterize this new damaging pathway, and test whether by targeting this pathway we can prevent blindness in animal models of LB-driven retinal disease."

www.brightfocus.org/grant/M2016124



Steven Nusinowitz, PhD (7/1/15 - 6/30/18)
Jules Stein Eye Institute, University of California, Los Angeles

Scotopic Critical Flicker Fusion in Preclinical AMD

"The main goal of this research project is to develop and test a novel method of evaluating retinal function in patients who are at risk of developing AMD, but who have not yet developed signs of the disease."

www.brightfocus.org/grant/M2015295

This grant is made possible in part by a bequest from the Anne E. Greene Trust.



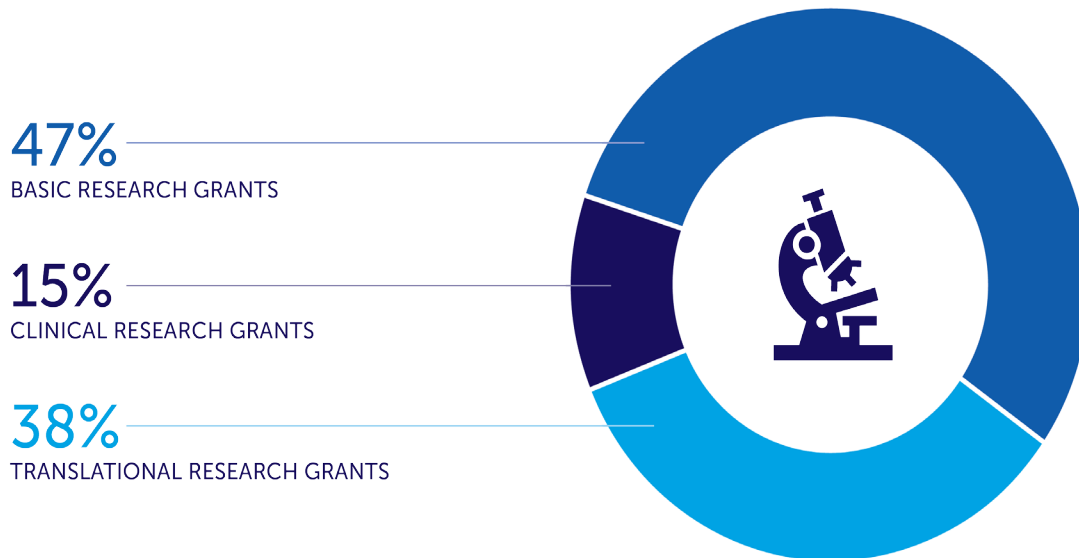
Debasish Sinha, PhD (7/1/16 - 6/30/18)
Wilmer Eye Institute, Johns Hopkins University, Baltimore, MD

Novel Therapeutic Targets for the Treatment of Early AMD

"Our proposed studies are aimed at developing novel small molecules that could be tested as a therapy for early AMD."

www.brightfocus.org/grant/M2016056

➤ BrightFocus Grants at a Glance



Basic - Research that aims to better understand how a disease happens, and to obtain new ideas of how to stop the disease.

Clinical - Research involving volunteer participants to test the safety and effectiveness of drugs, devices, or other treatment candidates.

Translational - Research to move findings from the lab bench to the "bedside" by testing potential treatments.

Special Focus on Geographic Atrophy

Geographic atrophy (GA) is a type of AMD that's also called "dry" AMD because it lacks the fragile, leaky blood vessels seen in late-stage "wet" AMD. Instead, photoreceptor cells weaken and die ("atrophy"), leading to an array of dead zones containing these atrophied cells that create blind spots at the center of the visual field. There currently is no treatment for late-stage GA. MDR is funding investigations into new drugs and ways to manage and treat this devastating disease, which could one day result in the first successful therapies.



Vera Bonilha, PhD (7/1/16 - 6/30/18)

The Cleveland Clinic Foundation, Ohio

Atrophic Lesion Borders in AMD: What Can They Tell Us?

"The information gained from this study will aid in understanding the pathophysiological mechanisms causally involved in GA and may offer additional insight in clinical diagnosis and therapeutic decision-making for GA."

www.brightfocus.org/grant/M2016079

Recipient of The Elizabeth Anderson Award



Jianhai Du, PhD (7/1/16 - 6/30/18)

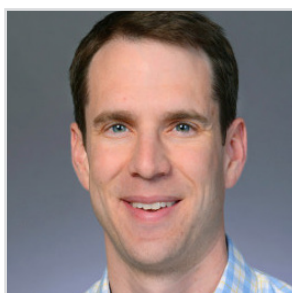
West Virginia University, Morgantown

A New Method to Decrease Cell Death by Supplementation with NAD Metabolites

"The goal of this project is to understand how energy metabolism is altered in AMD and test a nutritional approach to boost metabolism to prevent or rescue dry AMD."

www.brightfocus.org/grant/M2016047

This grant was made possible in part by the support from the Ivan Bowen Family Foundation.



John Hulleman, PhD (7/1/16 - 6/30/18)

The University of Texas Southwestern Medical Center, Dallas

A Single Genetic Manipulation for Treating Malattia Leventinese/ Dry AMD

"The goal of our project is to slow or prevent damage to a cell layer in the eye called the retinal pigment epithelium (RPE) by genetically reducing expression of a target protein in the retina that has been associated with inflammation and risk of macular degeneration."

www.brightfocus.org/grant/M2016200

This grant is made possible in part by a bequest from the Trust of Anne E. Greene.



Zhihong Hu, PhD (7/1/16 - 6/30/18)

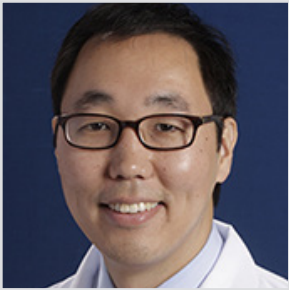
Doheny Eye Institute, University of California, Los Angeles

An Automated Method to Detect and Analyze Atrophic Lesions in AMD

"The deliverable from this research program is a fully automated system for the detection of geographic atrophy (GA) lesions and the quantitative analysis of GA progression."

www.brightfocus.org/grant/M2016088

This grant is made possible in part by a bequest from the Trust of Edna Stuver-Webster.



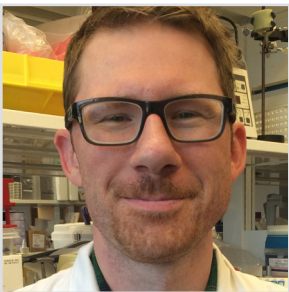
Benjamin Kim, MD (1/1/16 - 6/30/18)

University of Pennsylvania, Philadelphia

Evaluating Alpha Lipoic Acid as a Therapy for Geographic Atrophy

"The deliverable from this research program is a fully automated system for the detection of geographic atrophy (GA) lesions and the quantitative analysis of GA progression"

www.brightfocus.org/grant/CM2016971



Mikael Klingeborn, PhD (7/1/2015 - 6/30/18)

Duke University Eye Center, Durham, NC

The Role of Cell-Derived Lipid Vesicles in Early & Atrophic AMD

"Our research seeks to understand what causes the earliest stages of the dry form of AMD."

www.brightfocus.org/grant/M2015221



Regenerating Cells Damaged by AMD

Eyesight is precious and literally irreplaceable. Unlike skin and other parts of the human body, the nerve cells of the eyes do not, for the most part, regrow or regenerate after damage has occurred. However, there is hope, and work is moving forward to regenerate and reconnect the eye's retinal cells that have been damaged by age-related macular degeneration (AMD), and to restore the underlying RPE (retinal pigmented epithelium) that provides its nourishment and support. They are recreating parts of the eye using iPSC (induced pluripotent stem cell) technology (ie, stem cells derived from living adult tissue), and cell growth is being studied in other animal models with the hope of gleaning information that may benefit humans.



Jeffrey Gross, PhD (7/1/16 - 6/30/18)

University of Pittsburgh, Pennsylvania

Identification of Factors that Can Stimulate Regeneration of the RPE

"The goal of our work is to determine whether the human retinal pigment epithelium (RPE) can be stimulated to regenerate."

www.brightfocus.org/grant/M2016067



Biju Thomas, PhD (7/1/16 - 6/30/18)

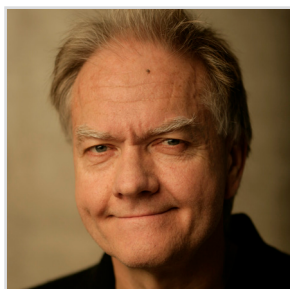
University of Southern California Roski Eye Institute, Los Angeles

Transplantation of iPS-RPE as a Polarized Monolayer

"We propose to treat such diseases by transplanting a polarized monolayer of retinal pigment epithelium (RPE) sheets derived from human induced pluripotent stem cells (iPS), which are stem cells that have been derived from adult human tissue."

www.brightfocus.org/grant/M2016186

This grant is made possible by a bequest from the Estate of Jane M. Simon.



Derek van der Kooy, PhD (7/1/16 - 6/30/18)

University of Toronto, Canada

Biomaterial-Based Stem Cell Therapies for Blinding Eye Disease

"We are focused on the goal of producing large quantities of cone photoreceptors for transplantation directly into the retina. Cones are the cells responsible for high-resolution/color vision and are lost in AMD."

www.brightfocus.org/grant/M2016173

This grant is made possible in part by a bequest from the Trust of Edward Primet.

Index:

Baird, Paul.....	7	Malek, Goldis.....	7
Baranov, Petr.....	10	McFarlane, Sarah.....	3
Bonilha, Vera.....	13	McGill, Trevor.....	9
Burns, Marie.....	5	Moreira, Ernesto.....	3
Canto-Soler, Maria Valeria.....	2	Mourrain, Philippe.....	8
Chao, Daniel.....	10	Nishina, Patsy.....	8
Chen, Jing.....	11	Nociari, Marcelo.....	11
Chen, Yan.....	2	Nusinowitz, Steven.....	12
Connor, Kip.....	11	Paulaitis, Michael.....	4
Daugherty, Patrick.....	5	Punzo, Claudio.....	4
Dinculescu, Astra.....	8	Rowan, Sheldon.....	9
Doyle, Sarah.....	5	Saban, Daniel.....	7
Du, Jianhai.....	13	Singh, Ruchira.....	3
Edwards, Malia.....	6	Sinha, Debasish.....	12
Esumi, Noriko.....	2	Thomas, Biju.....	15
Ghosh, Kaustabh.....	6	van der Kooy, Derek.....	15
Giorgianni, Francesco.....	6	Wang, Gaofeng.....	10
Gross, Jeffrey.....	15		
Guymer, Robyn.....	3		
Hauck, Stephanie.....	8		
Hu, Zhihong.....	14		
Hulleman, John.....	13		
Ildefonso, Cristhian.....	6		
Kim, Benjamin.....	14		
Klingeborn, Mikael.....	14		
Kumar-Singh, Rajendra.....	11		
Li, Binxing.....	9		

**Note: These are the active Macular Degeneration Research grants as of 7/20/2017.*



Macular Degeneration Research, a BrightFocus Foundation program

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